## Dr. Vito Imbasciani elected to serve as Chair of California stem cell agency

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Dr. Vito Imbasciani will be the new Chair of the California Institute for Regenerative Medicine (CIRM) , the state agency created by voters in 2004 and funded again in 2020 to invest in stem cell and regenerative medicine research and treatments.

At January's Board meeting, the agency's 35-member Governing Board elected Imbasciani to the six-year term, replacing outgoing chair Jonathan Thomas, who has served in the position since 2011.

"Dr. Imbasciani's experience across many relevant fronts will help him hit the ground running in guiding the Agency as it continues to grow its programs to bring treatments to patients with unmet medical needs," Thomas said in welcoming Imbasciani to the role. "The agency, as well as the people of California and the world, will be well served by Imbasciani's appointment as Chair of the CIRM Governing Board."

Imbasciani expressed excitement in taking on the role, citing his extensive career in academia, government, military service and medicine.

"My experience has positioned me to champion the aims of CIRM, advocate for it cogently, and represent it responsibly before the public and their state and federal elected representatives," Imbasciani said. "I look forward to the challenge of advancing the groundbreaking work of this Agency, at the same time nourishing the hopes for medical advances held by the citizens of our great State."

Imbasciani has served as the Secretary of the California Department of Veterans Affairs (CalVet) since 2015. As Secretary, he created several new programs within the department, including forging eight independent California veteran homes into a unified system, establishing programs for veterans in state prisons, and supporting the 58 county veteran service offices.

In addition, Dr. Imbasciani has been a practicing urologic surgeon for 30 years, treating a mostly older population suffering from congenital and acquired conditions.

Dr. Imbasciani completed medical school at the University of Vermont College of Medicine, and his surgical and urologic residencies at Yale-New Haven Hospital and the West Haven VA Hospital in Connecticut. At the University of Vermont, he worked in the laboratory assisting in studies of neurodegenerative diseases.

He earned MA and PhD degrees from Cornell University, and was a Fulbright Scholar to Rome, Italy in 1973. He held academic teaching positions at the University of Florida, Cornell University and Middlebury College in Vermont.

He also served for 27 years as a surgeon in the United States Army Medical Corps, with four wartime deployments that exposed him to battlefield medicine and post-acute care.

Dr. Imbasciani also has a documented history in successful stem cell research advocacy. As an elected member of the Board of Directors of both the California Medical Association and the Los Angeles County Medical Association, he advocated for investments in basic stem cell research, and for the passage of Proposition 71, the ballot initiative that created CIRM. This included participating in activities aimed at educating the wider medical community in the long-term benefits of stem cell research.

CIRM President and CEO Dr. Maria T. Millan applauded Imbasciani's appointment as Chair.

"Dr. Imbasciani's experience as a state secretary, surgeon, professor, stem cell research advocate, and board member of various medical agencies and organizations makes him exceptionally well-suited to fill the role of ICOC Chair and to lead CIRM in accelerating world class science and treatments for a diverse California and the world. I look forward to working with him in his new role."

Imbasciani will be sworn in and start on March 28, 2023.

The CIRM Board also awarded $15,457,858 for three projects in the agency's Clinical program. The goal of the Clinical program is to

speed up support for clinical stage candidate stem cell treatments that demonstrate scientific excellence.

Included in the Clinical awards is a $8 million grant to support Immusoft in a Phase I clinical trial to evaluate the safety and tolerability of a cell therapy for Mucopolysaccharidosis (MPS) I, a rare childhood genetic disease that causes organs and tissues of the body to become enlarged. The severe form of this disease is diagnosed at infancy and is fatal within the first 10 years of life.

This brings the number of CIRM-funded clinical trials to 87.

In this study, B cells will be isolated from patients suffering MPSI. These will be transformed with a normal copy of the gene and re-introduced into the patient.

Though there are approved treatments for MPSI, such as bone marrow transplantation, they have significant limitations. The proposed gene therapy for MPSI can potentially offer long-term disease control and prevent debilitating complications.

In addition, the Board awarded $4 million for a late-stage preclinical project by Ryne Bio aiming to improve treatment for Idiopathic Parkinson's disease (PD).

PD is the second-most common neurodegenerative disease after Alzheimer's disease affecting approximately 1 million people in the U.S. PD is characterized by a loss of dopamine producing neurons that result in motor symptoms, such as dyskinesias (involuntary, erratic, writhing movements of the face, arms, legs or trunk) and non-motor effects such as dementia, depression and sleep disorders.

Dopamine is a type of neurotransmitter and hormone that plays a role in many important body functions, including movement and memory.

In this proposed therapy, the goal is to deliver dopamine producing cells to replace the lost neurons to the brain of Parkinson's disease patients to restore/improve motor function.

The current grant is being funded to conduct Investigational New Drug (IND) enabling, nonclinical safety studies per the US Food and Drug Administration (FDA) Guidance. The IND is the authorization needed to begin a clinical trial in Parkinson's patients.

The list of successful applicants in CIRM's Clinical program are:

| Application | Program Title | Institution/Principal Investigator | Amount awarded |
|---|---|---|---|
| CLIN1-14070 | Development of cryopreserved interferon-gamma primed allogeneic MSCs, for treatment of steroid refractory acute graft versus host disease | Ossium Health - Brian Johnstone | $3,457,858 |
| CLIN1-14300 | Allogeneic iPSC derived Dopaminergic Drug Product for Parkinson's disease | Ryne Bio - Howard J Federoff | $4,000,000 |
| CLIN2-14416 | A Phase I Open Label Study to Evaluate the Safety and Tolerability of a Candidate in Patients with Mucopolysaccharidosis Type 1 | Immusoft Corporation - Robert Hayes | $8,000,000 |

The CIRM Board also awarded $32,992,265 to 16 projects in the agency's Quest Awards Program, which promotes the discovery of promising new stem cell-based and gene therapy technologies that could be translated to enable broad use and ultimately, improve patient care.

The successful applicants are:

| Application | Program Title | Institution/Principal Investigator | Amount awarded |
|---|---|---|---|
| DISC2-14130 | A Treatment for Artemis-deficient Severe Combined Immunodeficiency using Non-Viral CRISPR-driven Safe Harbor Transgenesis in Hematopoietic Stem Cells | Fyodor Urnov — University of California, Berkeley | $1,809,372 |
| DISC2-14190 | Reprogramming Somatic Cells into iPSCs Engineered with an Anti-PSCA CAR to Develop Allogeneic Off-the Shelf Cell Therapy to Treat Pancreatic Cancer | Jianhua Yu — Beckman Research Institute of City of Hope | $2,263,500 |
| DISC2-14049 | Microgel encapsulated iPSC-derived notochordal cells to treat intervertebral disc degeneration and low back pain | Dmitriy Sheyn — Cedars-Sinai Medical Center | $2,020,166 |
| DISC2-14180 | Excitatory spinal interneurons from human pluripotent stem cells to treat spinal cord injury | Lyandysha Viktorovna Zholudeva — The J. David Gladstone Institutes | $2,942,198 |
| DISC2-14045 | Novel Lipid Nanoparticles for Enhancing eNOS Synthesis for Cardioprotection Post Myocardial Infarction | Kevin E. Healy — University of California, Berkeley | $2,060,248 |
| DISC2-14090 | Gene Therapy for SLC6A8 Creatine Transporter Disorder | Gerald Lipshutz — University of California, Los Angeles | $2,296,920 |
| DISC2-14133 | Drug Discovery for Duchenne Muscular Dystrophy Using Patient-Derived Human iPSCs | Nikesh Kotecha — Greenstone Biosciences | $675,000 |
| DISC2-14187 | Expanded Capacity AAV Retinal Gene Therapy Enabled by Efficient RNA-Joining Technology | Lukas Bachmann — Vertuis Bio, Inc. | $1,446,000 |
| DISC2-14053 | Pluripotent Stem Cells for Tendon Tissue Engineering | Darryl D. D'Lima — Scripps Health | $2,734,163 |
| DISC2-14041 | Autologous stem cell-derived interneuron cell therapy for spinal cord injury (SCI) | Matthew Goodus — BrainXell Therapeutics | $2,025,000 |
| DISC2-14169 | Vax-T to promote formation of cancer-specific T memory stem cell for personalized cancer immunotherapy | Song Li — University of California, Los Angeles | $2,267,714 |
| DISC2-14083 | Development of novel small molecules against cancer stem cells in solid cancers | Frank Pajonk — University of California, Los Angeles | $2,340,000 |

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| DISC2-14096 | Pharmacological regenerative treatment of idiopathic pulmonary fibrosis targeting the senescent niche of lung progenitor cells. | Marco Quarta — Rubedo Life Sciences Inc. | $1,450,876 |
| DISC2-14166 | Reversal of dysregulated myelopoiesis in breast cancers and cancer stem cells to boost antitumor immunotherapy | Richard Joseph Pietras — University of California, Los Angeles | $2,327,680 |
| DISC2-14047 | A Novel Therapy for Sanfilippo B | Michelina Iacovino - Lundquist Institute | $2,297,884 |
| DISC2-14097 | In Utero Treatment of Duchenne Muscular Dystrophy with Non-viral Gene Editing | Aijun Wang – UC Davis | $2,035,544 |

**About CIRM**

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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